CYSTIC FIBROSIS FOUNDATION®

April 21, 2023

Dr. Lisa Ghotbi
Chief, Pharmacy Benefits Division
Department of Health Care Services
MS 4604 P. O. Box 997413
Sacramento, CA 95899-7413

Dear Dr. Ghotbi,

On behalf of the nearly 2,500 people with cystic fibrosis (CF) in California, we write regarding the state's forthcoming *Medi-Cal Rx Extended Duration Prior Authorization List*. We understand this list will identify certain medications used for chronic conditions that are eligible for extended duration/multi-year authorizations. As a chronic, genetic condition, people with CF take many therapies for their entire life and medication authorizations can present significant administrative burdens for patients and care teams. As the Department of Health Care Services finalizes the maintenance medication list, we recommend inclusion of the therapies listed below used for treatment of cystic fibrosis.

About Cystic Fibrosis
Cystic fibrosis is a life-threatening genetic disease that affects nearly 40,000 children and adults in the United States. CF causes the body to produce thick, sticky mucus that clogs the lungs and digestive system, which can lead to life-threatening infections. Cystic fibrosis is both serious and progressive; lung damage caused by infection is irreversible and can have a lasting impact on length and quality of life. As a complex, multi-system disease, CF requires an intensive treatment regimen including multiple medications. While these therapies are helping people with CF live longer, healthier lives, patients often encounter administrative barriers to accessing them.

Prior Authorizations for Medications Used to Treat Chronic Conditions
Prior authorizations are one obstacle all patients must navigate when getting care, but they present a unique set of challenges for people with CF and other chronic diseases with lifelong complex medical regimens. For people with CF, it is not uncommon to take seven therapies every day and as many as twenty.[1] Many of these medications are taken year after year, and in most cases, for life. Unnecessary prior authorizations can delay the start or continuation of needed treatments, leading to adverse health outcomes. Since CF is a progressive disease, interruptions in care put patients at risk of irreversible lung damage and costly hospitalizations.

Prior authorizations can also cause significant administrative burden for CF providers and are often redundant for medications that people with CF must take indefinitely to maintain their health. In a 2019 Cystic Fibrosis Foundation survey of over 100 CF care center directors, 60 percent cited the time and

[1] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC2680350/

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resources required for prior authorizations as one of the biggest barriers they face in supporting access to care and treatment. This process diverts valuable time and resources away from direct patient care.

As a result, we recommend that the state add CF therapies in the following drug classes to the *Medi-Cal Rx Extended Duration Prior Authorization List*.

CFTR modulator therapies correct the function of the defective protein made by the CF gene and have been transformative for eligible people with CF.

- Modulator therapies are available to people with specific CF variants and can preserve health and lung function and significantly slow the progression of the disease. For instance, recent data shows that treatment with elexacaftor/tezacaftor/ivacaftor results in sustained clinically meaningful improvements in lung function, respiratory symptoms, sweat chloride, and nutritional status at 144 weeks after initiating therapy.[2] These therapies are contributing to significant increases in life expectancy for people with CF as well, as data from the 2021 CF Foundation Patient Registry data shows that the anticipated survival for people with CF born between 2017 – 2021 is 53 years, compared to a survival of 43 for those born between 2012 – 2016.[3]

Mucus thinners, such as mucolytics, improve lung function and reduce infection rates by thinning the accumulated mucus and making it easier to cough out of the lungs.

- Airway clearance (of mucus) is an essential component of CF care. Treatment with mucolytic products has been shown to help clear mucus from the lungs, resulting in fewer lung infections, improved lung function, and a better quality of life for people with CF.[4,5,6,7]

Pancreatic enzymes help people with cystic fibrosis digest carbohydrates, proteins, and fats, and help with the absorption of vitamins and minerals.

- Approximately 90 percent of CF patients have pancreatic insufficiency, leading to malabsorption of calories and nutrients, and therefore, difficulty with growth and weight gain. Patients with pancreatic insufficiency require lifelong pancreatic enzyme replacement therapy (PERT) with each meal and snack to maintain adequate nutrition and prevent abdominal distress.
- PERT is a life-sustaining therapy for people with CF as nutritional status is closely linked to pulmonary function and survival.

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[2] Griese, M. et al. (2022). 170 Long-term safety and efficacy of elexacaftor/tezacaftor/ivacaftor in people with cystic fibrosis and at least one F508del allele: 144-week interim results from an open-label extension study. Journal of Cystic Fibrosis. 21. S99-S100. 10.1016/S1569- 1993(22)00861-X.

[3] 2021 Cystic Fibrosis Foundation Patient Registry Highlights Bethesda, Maryland ©2022 Cystic Fibrosis Foundation.

[4] Mogayzel, Peter, Jr., Naureckas, Edward, et al. Cystic Fibrosis Pulmonary Guidelines. American Journal of Respiratory and Critical Care Medicine, Vol. 187, 2013.

[5] Cramer, Gena W., et al. "The role of dornase alfa in the treatment of cystic fibrosis." *Annals of Pharmacotherapy* 30.6 (1996): 656-661.

[6] Elkins MR, Robinson M, Rose BR, et al. A controlled trial of long-term inhaled hypertonic saline in patients with cystic fibrosis. N Engl J Med 2006;354:229–240.

[7] Aitken ML, Bellon G, De Boeck K, et al. Long-term inhaled dry powder mannitol in cystic fibrosis: an international randomized study. Am J Respir Crit Care Med. 2012;185;6:645-52.

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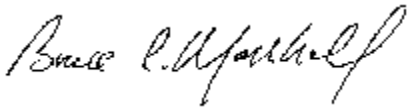
Inhaled antibiotics improve respiratory symptoms in people with cystic fibrosis who have *Pseudomonas aeruginosa*, a bacterium that colonizes in the lungs and is associated with increased morbidity and mortality in people with this disease.[8]

- *P. aeruginosa* infections can last for many years, resulting in many people with CF needing to chronically take inhaled antibiotics as a part of their maintenance treatment regimen.
- Use of CF specific antibiotics has been shown to decrease *P. aeruginosa* in sputum and improve lung function and quality of life.[9,10,11]

Anti-reflux medications such as proton pump inhibitors and histamine H2 receptor antagonists reduce stomach acid to prevent damage to the lining of the esophagus and stomach, and help improve the pH balance of the gastrointestinal tract so that PERT is more effective. People with CF have higher rates of gastroesophageal reflux disease as well as Barrett's esophagus and gastrointestinal malignancies to include esophageal cancer. Anti-reflux medications help mitigate these risks and are generally long-term medications.

We appreciate the Department's consideration of the needs of people with CF as they develop these policies for Medi-Cal Rx. Please contact Leslie Powell at lpowell@cff.org or (301) 215 - 7482 if you have questions.

Sincerely,



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[8] Emerson J, Rosenfeld M, McNamara S, Ramsey B, Gibson RL. *Pseudomonas aeruginosa* and other predictors of mortality and morbidity in young children with cystic fibrosis. *Pediatr Pulmonol* 2002;34:91-100.
[9] Retsch-Bogart GZ, Quittner AL, Gibson RL, Oermann CM, McCoy KS, Montgomery AB, Cooper PJ. Efficacy and safety of inhaled aztreonam lysine for airway *Pseudomonas* in cystic fibrosis. *Chest* 2009;135:1223-32.
[10] Ramsey BW, Pepe MS, Quan JM, Otto KL, Montgomery AB, Williams-Warren J, Vasiljev KM, Borowitz D, Bowman CM, Marshall BC*, et al.* Intermittent administration of inhaled tobramycin in patients with cystic fibrosis. Cystic Fibrosis Inhaled Tobramycin Study Group. *N Engl J Med* 1999;340:23-30.
[11] Quittner AL, Buu A. Effects of tobramycin solution for inhalation on global ratings of quality of life in patients with cystic fibrosis and *Pseudomonas aeruginosa* infection. *Pediatr Pulmonol* 2002;33:269-276.

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